

# SPECIALTY GUIDELINE MANAGEMENT

## ALDURAZYME (Iaronidase)

### POLICY

#### I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

##### FDA-Approved Indication

Aldurazyme is indicated for adult and pediatric patients with Hurler and Hurler-Scheie forms of Mucopolysaccharidosis I (MPS I) and for patients with the Scheie form who have moderate to severe symptoms.

##### Limitations of use:

- *The risks and benefits of treating mildly affected patients with the Scheie form have not been established.*
- *Aldurazyme has not been evaluated for effects on the central nervous system manifestations of the disorder.*

All other indications are considered experimental/investigational and not medically necessary.

#### II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

- A. Initial requests: alpha-L-iduronidase enzyme assay and/or genetic testing results supporting diagnosis.
- B. Continuation requests: chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

#### III. CRITERIA FOR INITIAL APPROVAL

##### **Mucopolysaccharidosis I (MPS I)**

Authorization of 12 months may be granted for treatment of MPS I when both of the following criteria are met:

- A. Diagnosis of MPS I was confirmed by enzyme assay demonstrating a deficiency of alpha-L-iduronidase enzyme activity and/or by genetic testing.
- B. Member has one of the following:
  1. The Hurler form (i.e., severe MPS I).
  2. The Hurler-Scheie form (i.e., attenuated MPS I).
  3. The Scheie form (Scheie syndrome; i.e., attenuated MPS I) with moderate to severe symptoms (e.g., normal intelligence, less progressive physical problems, corneal clouding, joint stiffness, valvular heart disease).

#### IV. CONTINUATION OF THERAPY

<b>Reference number(s)</b>
2049-A

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III who have a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

## V. REFERENCES

1. Aldurazyme [package insert]. Cambridge, MA: Genzyme Corporation; March 2023.
2. Wraith JE, Clarke LA, Beck M, et al. Enzyme replacement therapy for mucopolysaccharidosis I: a randomized, double-blinded, placebo-controlled, multinational study of recombinant human alpha-L-iduronidase (laronidase). *J Pediatr.* 2004;144:581-588.
3. Muenzer J, Wraith JE, Clarke LA; International Consensus Panel on Management and Treatment of Mucopolysaccharidosis I. Mucopolysaccharidosis I: management and treatment guidelines. *Pediatrics.* 2009 Jan;123(1):19-29.
4. Clarke LA. Mucopolysaccharidosis Type I. 2002 Oct 31 [Updated 2021 Feb 25]. In: Adam MP, Everman DB, Mirzaa GM, et al., editors. *GeneReviews®* [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2023. Accessed Jan 3, 2024